
# Assessment of Barriers to Fair Access

**Final Report**

**January 17, 2023**

**Institute for Clinical and Economic Review**

**AUTHORS**:

**Steven D. Pearson, MD, MSc**
President
Institute for Clinical and Economic Review

**Matt Seidner, BS**
Director of Policy Implementation
Institute for Clinical and Economic Review

**Molly Beinfeld, MPH**
Former Senior Research Lead, Evidence Synthesis
Institute for Clinical and Economic Review

**Belén Herce-Hagiwara, BA**
Research Assistant
Institute for Clinical and Economic Review

**Serina Herron-Smith, BA**
Associate Research Manager
Institute for Clinical and Economic Review

**Victoria Lancaster, PharmD, MSc, MBA**
Former Health Technology Assessment Fellow
Institute for Clinical and Economic Review

**Max Lee, PharmD**
Pharmaceutical Intelligence Manager
Institute for Clinical and Economic Review

**Maggie O'Grady, BS**
Former Program Manager
Institute for Clinical and Economic Review

**Marina Richardson, MSc**
Senior Health Economist
Institute for Clinical and Economic Review

**Yvette Venable**
Former Vice President of Patient Engagement
Institute for Clinical and Economic Review

**Sarah K. Emond, MPP**
Executive Vice President and Chief Operating Officer
Institute for Clinical and Economic Review

We would also like to thank Meaghan Cummings and Grace Lin for their contributions to this report.

**DATE OF PUBLICATION:** January 17, 2023

**How to cite this document:** Pearson SD, Seidner M, Beinfeld M, Herce-Hagiwara B, Herron-Smith S, Lancaster V, Lee M, O'Grady M, Richardson M, Venable Y, Emond SK. Assessment of Barriers to Fair Access. Institute for Clinical and Economic Review, January 17, 2023. https://icer.org/policy-papers/fair-access-2022/

## About ICER

The Institute for Clinical and Economic Review (ICER) is an independent non-profit research organization that evaluates medical evidence and convenes public deliberative bodies to help stakeholders interpret and apply evidence to improve patient outcomes and control costs. Through all its work, ICER seeks to help create a future in which collaborative efforts to move evidence into action provide the foundation for a more effective, efficient, and just health care system. More information about ICER is available at https://icer.org/.

# Table of Contents

# List of Acronyms and Abbreviations Used in this Report

| | |
|---|---|
| **ACA** | Affordable Care Act |
| **AHRQ** | Agency for Healthcare Research and Quality |
| **AMA** | American Medical Association |
| **BCBS** | Blue Cross Blue Shield |
| **BCBS MA** | Blue Cross Blue Shield of Massachusetts |
| **BCBS MI** | Blue Cross Blue Shield of Michigan |
| **Blue Shield CA** | Blue Shield of California |
| **CGRP** | Calcitonin gene-related peptide |
| **evLYG** | Equal value of life years gained |
| **FDA** | U.S. Food and Drug Administration |
| **FSS** | Federal Supply Schedule |
| **HBPB** | Health benefit price benchmark |
| **HCSC** | Health Care Service Corporation |
| **HIX** | Health Insurance Exchange |
| **ICER** | Institute for Clinical and Economic Review |
| **IRS** | Internal Revenue Service |
| **Mg** | Milligram |
| **PBM** | Pharmacy benefit manager |
| **QALY** | Quality-adjusted life year |
| **UC** | Ulcerative colitis |
| **US** | United States |
| **VHA** | Veterans Health Administration |

# Executive Summary

The national debate about drug pricing has focused attention on methods to determine whether the price of a drug is "fair" or "reasonable." A question far less examined is how to determine whether insurance coverage is providing fair access to that drug. To help address these questions, ICER developed a set of appropriateness criteria for pharmaceutical insurance coverage, as described in our 2020 white paper, Cornerstones of "Fair" Drug Coverage: Appropriate Cost-Sharing and Utilization Management Policies for Pharmaceuticals.

In this paper, we apply several key criteria from the white paper to the real-world coverage policies for 19 drugs reviewed by ICER in 2020 (Nurtec, Ubrelvy, and Reyvow for acute migraine; Hemlibra for hemophilia A; Kalydeco, Orkambi, Symdeko, and Trikafta for cystic fibrosis; Inflectra, Remicade, Renflexis, Entyvio, Humira, Simponi, Stelara, and Xeljanz for ulcerative colitis; Adakveo, Endari, and Oxbryta for sickle cell disease). We sought coverage policies for these drugs from 18 formularies, including the 15 largest commercial formularies in the United States, the formulary of the Veteran's Health Administration (VHA), and the formularies of the two largest state Affordable Care Act (ACA) exchange plans. We leveraged the MMIT Analytics Market Access Database as the primary source of policy information and invited payers to provide policies when documents were not available in the database. At the time we conducted our search, these formularies represented coverage policies governing pharmaceutical access for approximately 55 million Americans.

We rated the concordance of the coverage policies against specific elements of ICER's broader fair access criteria for 1) cost sharing to patients; 2) clinical eligibility criteria; 3) step therapy and required switching policies; and 4) provider qualification restrictions. We also conducted two exploratory analyses of whether payers provide sufficient transparency into clinical eligibility criteria and cost sharing requirements for individuals comparing health insurance policies, and an analysis of the burden created by the number of prior authorization questions that providers must complete for patients to gain access to their desired drug. These exploratory analyses applied only to drugs for acute migraine and ulcerative colitis (UC).

Overall rates of concordance with ICER's criteria were 70% for cost sharing of fairly-priced drugs, 96% for clinical eligibility criteria, 98% for step therapy, and 100% for provider restrictions. In the exploratory transparency analysis for select migraine and UC drugs, 16/18 (89%) of payers made tiering information available. Adequate information on clinical coverage criteria were provided by 9/13 (64%) of health insurers and 1/5 (20%) of pharmacy benefit managers. In the exploratory analysis of prior authorization documentation burden, the forms requiring provider input had median numbers of questions that ranged from 25 to 36 across the set of migraine and UC drugs, with the full range of the number of questions across payer forms extending from 22 to 71.

Payers were given several weeks to provide comments on a draft of this assessment. As part of their feedback, five payers informed us that they had revised their coverage policies on a total of 11 drugs in ways that would move coverage into concordance with our fair access criteria.

There are several important limitations to these findings. First, we were unable to assess many important fair access criteria, including whether patient cost sharing is based on the plan's negotiated price for a drug rather than the drug's list price. Formulary tier placement as a surrogate for cost sharing is an imperfect analogue for cost sharing since specific cost sharing amounts and the choice of co-pay versus co-insurance are decided by the plan sponsor and not the payer. We were also unable to assess whether payers administered their policies (e.g., the process for requesting exceptions to medical coverage criteria) in line with our fair access criteria. The data we used to determine net prices are an average across all payers and included patient financial assistance, so we were unable to determine whether any individual payer's negotiated price was in line with ICER's estimates of a reasonable price. Finally, it is possible that the formularies selected for this assessment provide superior coverage than formularies from the same payers or other companies that cover fewer individuals.

While the evidence available and the limitations of our research effort leave many questions, our results demonstrate that the great majority of payer policies in the formularies evaluated are structured in a way to support many key elements of fair access. In addition, the changes in coverage policies noted following initial assessment show that payers are open to reflection regarding their policies and that collaborative efforts to hold payers accountable may lead to positive change. However, important work remains to be done. We found that many payers do not provide adequate transparency into clinical coverage criteria, creating barriers for individuals seeking to understand whether joining or switching to a new payer would leave them at risk of losing coverage for an important medication. Our analysis also found that prior authorization forms can run to dozens and dozens of questions, raising the risk that minor errors or lack of information could block coverage and lead to delays in receiving appropriate care. Reinforcing the results of broad surveys of providers and patients, we also received input from patient organizations presenting stark examples of patients experiencing barriers to fair access arising from what can be labyrinthine and prolonged procedures that undermine the purported goal of appropriate access.

This report therefore can have no simple conclusion on the degree of fair access to medications across the drugs and payers evaluated. Instead, we hope it will serve to foster further collaborative efforts to define the parameters of fair access and to work to elevate these ideals as a pillar of a just health care system.

# 1. Introduction

The national debate about drug pricing has focused attention on methods to determine whether the price of a drug is "fair" or "reasonable." A question far less examined is how to determine whether insurance coverage is providing fair access to that drug. It is widely agreed that cost sharing and drug coverage criteria serve everyone's interest when they steer patients toward evidence-based use of treatments that achieve equal or better outcomes at lower costs. But this level of conceptual agreement does little to help advance thinking on how to assess and judge specific cost-sharing provisions and prior authorization protocols. Is it fair to have patients pay at the highest cost-sharing level when there is only a single drug available in a drug class? What are the circumstances under which step therapy is a reasonable approach? When is it appropriate for the clinical criteria for coverage to be narrower than the Food and Drug Administration (FDA) labeled indication? And how should whether a drug is priced reasonably or not affect judgments of the appropriateness of certain strategies to manage its utilization?

To help address these questions, ICER developed a set of appropriateness criteria for pharmaceutical insurance coverage, as described in our 2020 white paper, Cornerstones of "Fair" Drug Coverage: Appropriate Cost-Sharing and Utilization Management Policies for Pharmaceuticals. Readers of this current assessment are encouraged to read the earlier white paper to understand the broader ethical analysis and stakeholder input that were the foundation for these appropriateness criteria. This process featured a December 2019 ICER Policy Summit attended by representatives from patient groups, clinical specialty societies, private payers, and the life sciences industry.

The goal of this larger initiative from the outset has been for the "Fair Access" criteria to serve as a tool for assessment and as the starting point for dialogue and action to achieve fair access. In 2021, ICER applied a subset of the criteria to the coverage policies of leading commercial payers in our first Barriers to Fair Access Assessment. Based on the experience with the first report, and with ongoing input from our multi-stakeholder Working Group, we have modified our methods for the 2022 report to expand the scope of fair access criteria to be evaluated, increase the number of payer formularies in the assessment, and shift the process for identifying the set of the drugs whose coverage policies will be evaluated so that it includes all drugs reviewed by ICER during the calendar year two years prior.

The goals and basic approach of this 2022 report remain consistent with those of the 2021 report. We have continued to leverage the MMIT Analytics Market Access Database as the primary source of coverage policy information. This year we are assessing coverage for the 19 drugs reviewed by ICER in 2020, across 18 formularies, including the 15 largest commercial formularies in the United States, the formulary of the Veterans Health Administration (VHA), and the formularies of the two largest state Affordable Care Act (ACA) exchange health plans. One payer, Kaiser Foundation Health Plans, appears twice in our analysis – once for its commercial formulary, and once for its state exchange

formulary. For each of the 19 drugs, we have performed analyses of the proportion of selected fair access criteria that are met in the coverage policies within these formularies.

As noted earlier, for the 2022 report, we have added several additional fair access criteria and have updated a key aspect of our step therapy criteria. Specifically, we have evaluated concordance on a select set of drugs and formularies on criteria related to the **relative burden of prior authorization** and the **transparency of cost sharing and clinical eligibility criteria** to prospective plan enrollees. We have also added a maximum number of three steps allowed for a step therapy policy to remain concordant with fair access criteria, even if there are more than three potential steps that could be considered to include appropriate first-line therapies. This represents a change from the original fair access criteria, which did not specify a maximum number of steps. More detailed explanation of these methods changes is provided in the body of the report and Supplemental Material.

The key limitations of this analysis will be emphasized throughout the report and are summarized in Table 1 below. First, among the full set of fair access criteria contained in the white paper, many were not able to be assessed given that they cannot be determined from viewing insurance coverage and tiering information. All of these criteria aim to lessen patient financial burden or represent standards for the use of evidence in framing access restrictions, thus our inability to assess them reduces our ability to frame a comprehensive judgment of whether payers are meeting fair access criteria. Second, while we were able to assess the relative level of documentary burden for prior authorization policies, we were not able to assess the ease of obtaining reasonable exceptions, which is critical to achieving fair access. Third, for judgments on cost sharing, we could only use tiering as a signal of the relative magnitude of out-of-pocket payment required, an approach that does not capture the wide variety of levels of co-payments and co-insurance that can be used by plan sponsors within any tiering structure. And fourth, our selection of the commercial formularies with the largest number of covered lives for each payer may skew our analysis toward formularies with policies more, or less, in concordance with the fair access criteria than a broader sample of American payers.

**Table 1. Key Limitations to This Analysis of Barriers to Fair Access**

| Key Limitations |
|---|
| 1. There are many important fair access design criteria not able to be evaluated from insurance coverage policies alone, including, for example:<br>a. Patient cost sharing should be based on the net price to the plan sponsor, not the unnegotiated list price;<br>b. As part of step therapy, when patients try a lower cost option with a lower cost-sharing level but do not achieve an adequate clinical response, cost sharing for further therapies should also be at the lower cost-sharing level if those further therapies are priced fairly;<br>c. Clinical eligibility criteria should be developed with explicit mechanisms that require payer staff to document that they have confirmed that clinical eligibility criteria have not gone beyond reasonable use of clinical trial inclusion/exclusion criteria to interpret or narrow the FDA label language in a way that disadvantages patients with underlying disabilities unrelated to the condition being treated |

| | |
|---|---|
| 2. | We were unable to assess the efficiency of the process for requesting and adjudicating medical exceptions for individual patients. |
| 3. | Tiering as a surrogate for cost sharing is not able to reflect the actual out of pocket cost sharing amount nor whether co-payment versus co-insurance is required |
| 4. | The data used to determine drug prices net of rebates are an average across all payers, including 340B institutions, and calculations include patient co-payment assistance and other expenditures that do not flow back to payers as rebates; therefore for any individual payer the net price they pay for a drug may not align precisely with our data, creating a risk for heterogeneity across payers in whether drugs have a "cost-effective" price and thus require preferential tiering to meet fair access criteria |
| 5. | It is possible that the 15 commercial formularies selected for this assessment provide superior coverage than formularies covering fewer individuals offered by the same payer |

FDA: U.S. Food and Drug Administration

To help provide important guidance on this project, the Barriers to Fair Access Assessment has benefited from ongoing input from a multi-stakeholder Working Group consisting of several representatives from leading patient advocacy groups, two from clinical specialty societies, one from a pharmacy benefit manager, and one from an umbrella organization for life sciences companies. The Working Group has advised ICER on the application of the fair access criteria to coverage policies; provided insight into the patient experience with prescription drug coverage and access, including real-world examples; and advised on important nuances in the interpretation of payer coverage policies. None of them should be assumed to agree with any of the specific methods, findings, or perspectives presented in this report. Members of the Working Group are listed in the Supplemental Material.

# 2. Drugs and Formularies to be Assessed

The 19 drugs reviewed by ICER in 2020 are shown in Table 2 below. Average net prices between January 2021 and December 2021 were calculated based on data from SSR Health, LLC, an independent investment research firm. SSR Health estimates net price by calculating sales revenue net of all discounts, rebates, concessions to wholesalers and distributors, and the costs of patient assistance programs, and dividing this revenue by unit sales data. Table 2 below divides the list of drugs into those with net prices from SSR Health that fall below $150,000 per quality-adjusted life year (QALY) or equal value of life years gained (evLYG), and are therefore considered to be fairly priced at a cost-effective level, and those above that threshold.

**Table 2. Drug List**

| Brand Drug Name | Generic Drug Name | Condition | Maximum Annualized Cost-effective Price* |
|---|---|---|---|
| **Drugs With Net Prices at or Below $150,000 per QALY or evLYG†** | | | |
| Hemlibra® | Emicizumab | Hemophilia A | Cost saving |
| Inflectra® | Infliximab-dyyb | Ulcerative Colitis | $11,034 |
| Nurtec® | Rimegepant | Migraine: Acute | $4,697 |
| Remicade® | Infliximab | Ulcerative Colitis | $11,034 |
| Renflexis® | Infliximab-abda | Ulcerative Colitis | $11,034 |
| Ubrelvy™ | Ubrogepant | Migraine: Acute | $4,687 |
| **Drugs With Net Prices Above $150,000 per QALY or evLYG†** | | | |
| Adakveo® | Crizanlizumab | Sickle Cell Disease | $35,046 |
| Endari® | L-glutamine | Sickle Cell Disease | $19,568 |
| Entyvio® | Vedolizumab | Ulcerative Colitis | $11,844 |
| Kalydeco® | Ivacaftor | Cystic Fibrosis | $74,303 |
| Humira® | Adalimumab | Ulcerative Colitis | $6,985 |
| Orkambi® | Lumacaftor/Ivacaftor | Cystic Fibrosis | $61,750 |
| Oxbryta® | Voxelotor | Sickle Cell Disease | $23,668 |
| Reyvow ® | Lasmiditan | Migraine: Acute | $3,189 |
| Simponi® | Golimumab | Ulcerative Colitis | $7,693 |
| Symdeko® | Tezacaftor/Ivacaftor | Cystic Fibrosis | $70,760 |
| Stelara® | Ustekinumab | Ulcerative Colitis | $16,804 |
| Trikafta® | Elexacaftor/Tezacaftor/Ivacaftor | Cystic Fibrosis | $86,552 |
| Xeljanz® | Tofacitinib | Ulcerative Colitis | $15,488 |

evLYG: Equal value of life years gained, QALY: quality-adjusted life year

*For details on dosing and pricing assumptions please see ICER Reports or ICER Analytics

† The higher of the $150,000 per evLYG or QALY threshold price was considered the maximum cost-effective price.

For these 19 drugs we used MMIT's Market Access Analytics platform to obtain cost sharing and prior authorization documentation from the relevant payer formularies. Prior to the initiation of this assessment, we evaluated the relative strengths of different academic and commercial databases detailing insurer coverage policies and found MMIT's platform to be the most comprehensive available. Details on how MMIT assigns who "controls" a formulary and the covered lives under each formulary are provided in the Supplement. The formularies marketed under Express Scripts and Cigna Corporation remain separate in this database even though the companies are now merged because each company continues to make formulary decisions independently. Formularies for OptumRx and UnitedHealthcare are also evaluated separately because even though they use the same underlying template, UnitedHealthcare has the discretion to design its own coverage policies, which can differ from those in the OptumRx formulary.

Together, these formularies represent coverage policies governing pharmaceutical access for approximately 55 million Americans (MMIT Analytics as of 07/14/2022). See Table A4.1 in the Supplement for detailed information on covered lives per formulary.

**Table 3. Largest Single Formulary Offered by Each of the 15 Largest Commercial Payers, the VHA, and the Two Largest State Exchanges, Ordered by Number of Covered Lives***

| Payer | Formulary Name | Plan Type | Tiers Available |
|---|---|---|---|
| CVS Health | CVS Caremark Standard Control w/ Advanced Specialty Control | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand |
| Express Scripts | Express Scripts National Preferred with Advantage Plus | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand |
| UnitedHealthcare | UnitedHealthcare Advantage Three Tier | Commercial | Tier 1: Lowest cost<br>Tier 2: Mid-range cost<br>Tier 3: Highest cost |
| Department of Veterans Affairs | VHA National Formulary | Federal | Not applicable |
| Cigna Corporation | Cigna Standard Three Tier | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand |
| OptumRx | OptumRx Select Standard | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand |
| Kaiser Foundation Health Plans, Inc. | Kaiser Permanente Southern California | Commercial | Tier 1: Generic<br>Tier 2: Brand |
| Anthem | Anthem Essential Four Tier | Commercial | Tier 1: Preferred Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand<br>Tier 4: Specialty |
| Blue Cross Blue Shield (BCBS) of Massachusetts | BCBS Massachusetts Three Tier | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand |

| Payer | Formulary Name | Plan Type | Tiers Available |
|---|---|---|---|
| Health Care Service Corporation (HCSC) | BCBS of Illinois Basic 6 Tier | Commercial | Tier 1: Preferred Generic<br>Tier 2: Non-Preferred Generic<br>Tier 3: Preferred Brand<br>Tier 4: Non-Preferred Brand<br>Tier 5: Preferred Specialty<br>Tier 6: Non-Preferred Specialty |
| Elixir PBM | Elixir Standard Formulary | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Brand |
| MedImpact Healthcare Systems, Inc | MedImpact Portfolio High Formulary | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Brand |
| Highmark, Inc. | Highmark Blue Cross Blue Shield 3 Tier | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Generic or Non-Preferred Brand |
| Blue Shield of California | Blue Shield of California Plus Formulary | Commercial | Tier 1: Preferred Generic or Low-Cost Preferred Brand<br>Tier 2: Non-Preferred Generic or Preferred Brand<br>Tier 3: Non- Preferred Brand<br>Tier 4: Biologics or Specialty |
| Florida Blue | Florida Blue Care Choices HIX | State Exchange | Tier 1: Preventive<br>Tier 2: Condition Care Generic<br>Tier 3: Other Generic<br>Tier 4: Condition Care Brand<br>Tier 5: Preferred Brand<br>Tier 6: Non-Preferred Brand<br>Tier 7: Specialty |
| Premera Blue Cross | Premera Preferred 3-Tier-B3 | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Brand |
| Kaiser Foundation Health Plans, Inc. | Kaiser Permanente California HIX | State Exchange | Tier 1: Generic<br>Tier 2: Brand<br>Tier 4: Specialty |
| Blue Cross Blue Shield of Michigan | BCBS Michigan Custom 3 Tier | Commercial | Tier 1: Generic<br>Tier 2: Preferred Brand<br>Tier 3: Non-Preferred Brand |

*Covered lives as of 07/14/2022 according to MMIT

# 3. Fair Access Criteria

There are many potential barriers to access spanning health literacy, disability status, provider education and availability, personal resources, and access to affordable insurance coverage. ICER's original white paper focused narrowly on two areas over which plan sponsors and payers (inclusive of both pharmacy benefit managers [PBMs] and insurers) have the most control: cost-sharing provisions and the design and implementation of utilization management.

Given this focus, the white paper did not address many other important areas of coverage policy, including thresholds for the number or type of drugs needed within drug classes; coverage for off-label prescribing; potential changes to the current rebate system; high-deductible benefit designs; and the role of co-payment coupons. Instead, the fair access criteria in the white paper directly address the following five domains:

- Cost-sharing provisions and tier placement as part of the drug benefit design
- Timing of development of prior authorization protocols following FDA approval
- Clinical eligibility criteria
- Step therapy and coverage requirements to switch medications
- Restrictions on prescriber qualifications

The purpose of the current assessment was to evaluate concordance of payer coverage policies for the 19 drugs that were the subject of ICER evidence reviews in 2020 and are currently FDA approved for the indication for which ICER evaluated them. In designing this assessment, we had to make several important concessions. First, we felt we would not have the time or resources to be able to do a separate investigation with each payer to seek permission to obtain and evaluate their coverage policies. Instead, we began by leveraging the MMIT Analytics Market Access Database for formulary information and supplemented this database by asking payers to update any information that was no longer current and to fill in any gaps of information in the MMIT database. Second, we had to acknowledge that some of the fair access criteria would not be able to be evaluated without site visits, in-depth interviews, or access to material related to implementation of coverage policy procedures. Given our available resources, and the length of time it would take to perform a full, in-depth assessment of implementation, we decided not to evaluate the timing of development of prior authorization following FDA approval and several other important elements of fair access, such as responsiveness to initial requests for coverage, or timeliness of responses to requests for medical exceptions. We will stress throughout this report that these elements of fair access are critically important to patient and clinician experience and to patient outcomes. The fact that this current assessment did not evaluate these factors should be viewed as an important limitation on generalizing any judgment of whether a particular coverage policy represents "fair access."

As noted earlier, for the 2022 report we have included a new element for our step therapy fair access criteria. Based on a recent analysis of step therapy protocols,[1] we introduced a threshold of a maximum of 3 steps for any policy, meaning that any step therapy policy requiring 4 or more steps to get to a particular covered drug, even if each step in isolation was judged clinically appropriate, was determined to have failed to meet fair access criteria for step therapy.

In this year's report we have also added an exploratory evaluation of concordance with fair access criteria in two important areas not assessed in 2021: 1) the transparency to prospective plan enrollees of cost sharing (i.e., tiering) and of clinical eligibility criteria; and 2) prior authorization documentation burden. Given that we have not attempted to evaluate these domains of fair access before, we chose to perform a targeted evaluation of coverage policies limited to the migraine and ulcerative colitis treatments in the data set.

We present below and on the following pages the entire set of fair access criteria from the original white paper, indicating which criteria we have been able to include within the scope of this current assessment.

**Table 4. Cost Sharing Fair Design Criteria**

| **Cost Sharing** | |
|---|---|
| **Fair Design Criteria** | **In Scope for this Review?** |
| Patient cost sharing should be based on the net price to the plan sponsor, not the unnegotiated list price. | No |
| All medications identified by the Internal Revenue Service as high-value therapies should receive pre-deductible coverage within high deductible health plans. | No |
| At least one drug in every class should be covered at the *lowest relevant* cost-sharing level unless all drugs are priced higher than an established fair value threshold. | Yes |
| If all drugs in a class are priced so that there is not a single drug that represents a fair value as determined through value assessment, it is reasonable for payers to have all drugs on a higher cost-sharing level. | Yes |
| If all drugs in a class are priced so that they represent a fair value, it remains reasonable for payers to use preferential formulary placement with tiered cost sharing to help achieve lower overall costs. | Yes |
| As part of economic step therapy, when patients try a lower cost option with a lower cost-sharing level but do not achieve an adequate clinical response, cost sharing for further therapies should also be at the lower cost-sharing level as long as those further therapies are priced fairly according to transparent criteria. | No |

*Commentary on Assessment Strategy for Cost Sharing*

We have evaluated cost-sharing concordance only on the basis of the tiering of a drug within the pharmacy benefit, even for those payers who provide coverage under both pharmacy and medical benefits. The rationale for this approach is that formulary tiers often do not exist within medical benefit designs, and cost sharing within the medical benefit is determined by plan sponsors. While we heard from payers that claims under the medical benefit may represent the vast majority of

claims for certain drugs covered under both benefits, we felt it was important to evaluate cost sharing under the pharmacy benefit because it is under the control of the payer and should meet fair access criteria even if a very small number of patients are affected.

To meet the criterion for cost sharing, a fairly-priced drug or at least one of its equivalent options must be placed on the "lowest relevant" tier of the formulary. The interpretation of which is the lowest relevant tier for certain drugs is made difficult by the number and labeling of tiers in different formularies. For the purposes of this report, we required a fairly-priced drug to be placed in the second tier ("preferred brand") for formularies built with three or four tiers. Thus, even for four-tier formularies with a single "specialty" fourth tier, the formulary was required to place these drugs on the second tier in order to be judged concordant.

This approach was informed by input from payers who noted that they ultimately have discretion on whether to place an expensive "specialty" drug on a lower tier. However, payers also noted that four-tier formularies are designed in conjunction with plan sponsors and that a payer has an implied responsibility to administer a four-tier formulary by putting all specialty drugs on the fourth tier. In addition, some plan sponsors may choose to have the same cost-sharing amount for drugs on a specialty fourth tier as on a preferred brand tier; and even if the amount is higher, it may still be a co-payment amount that is less than the amount required out of pocket for the same drug on the third tier of some three-tier formularies from other payers.

The difficulty in interpreting tiering level as a surrogate for cost sharing is compounded by the way tiered formularies are related to high-deductible health benefit designs. As shown in a report from Kaiser Family Foundation, in 2020, a higher percentage of all employees in plans without high deductibles had plans with four or more tiers (54%) than three tiers (35%). The report also noted that, whereas the percentage of four-tier formularies requiring co-insurance is higher than that for three-tier formularies (36% to 24%), most four-tier formularies still require only co-payments for all tiers.[2]

Thus, the correlation of tiering level and actual out-of-pocket cost is not exact across formularies. Our approach to evaluating tier placement emphasizes a judgment about relative cost sharing rather than absolute cost sharing, and the actual question of whether cost sharing is presenting an unfair barrier to access can only be answered at the level of the individual plan sponsor.

The existence of manufacturer coupons and other patient assistance programs further complicates the assessment of patient out-of-pocket costs. Manufacturer coupons and patient assistance programs defray some or all of the co-payment or co-insurance for a prescription and, when allowed, contribute toward deductibles and annual out of-pocket maximums. When no generic alternatives are available, these programs shield patients from the rising costs of branded drugs. However, while undoubtedly beneficial for individuals, these programs have been criticized for encouraging patients

to take more expensive branded drugs when cheaper options are available, increasing plan spending and ultimately increasing the costs of pharmaceutical coverage.[3]

Benefit designs using either co-pay maximizer or accumulator mechanisms represent another important limitation in our ability to use formulary tiering as a surrogate for the cost sharing requirements for patients. Under co-pay accumulators, co-pay coupons and patient assistance programs are not applied to deductibles or out of pocket spending; patients must use the maximum amount of assistance for which they are eligible, after which they must meet their deductible and out-of-pocket spending obligations. This approach can subject patients to high out-of-pocket costs, sometimes even in cases where no cheaper generic alternatives are available. In contrast, maximizers set the patient's out of pocket expenses for the drug to the maximum amount of the assistance program and ensures those costs are spread over the full year. Maximizers typically shield patients from high out-of-pocket costs at the same time they increase manufacturer payments to insurers. Both of these programs, which are intended in part to address the aforementioned dynamic that incentivizes brand medications over less expensive alternatives, can serve more as a mechanism to reduce plan costs beyond what they would be even without the application of co-payment coupons or manufacturer assistance. Co-pay accumulators, in particular, can increase patients' cost burdens, especially in conditions for which there are no alternatives to branded medications.[4]

Despite these limitations in using tiering as an indicator of a fair approach to cost sharing, we believe that the general principle still holds: fairly-priced drugs should be placed on the lowest available relevant tier, which for brand name drugs is the second (preferred brand) tier. When results are presented in a later section for each payer formulary, the distinction between the relative concordance rates for four-tier formularies versus other formulary designs will be evident. One of the main points of further discussion should be whether four-tier formularies are structurally more likely to represent a barrier to fair access or whether they should be held to a different standard than other formulary designs.

**Table 5. Clinical Eligibility Fair Design Criteria**

| Clinical Eligibility | |
|---|---|
| **Fair Design Criteria** | **In Scope for this Review?** |
| Payers should offer alternatives to prior authorization protocols such as programs that give feedback on prescribing patterns to clinicians or exempt them from prior authorization requirements ("gold carding") if they demonstrate high fidelity to evidence-based prescribing. | No |
| Payers should document at least once annually that clinical eligibility criteria are based on high quality, up-to date evidence, with input from clinicians with experience in the same or similar clinical specialty. | No |
| Clinical eligibility criteria should be developed with explicit mechanisms that require payer staff to document that they have:<br>• Considered limitations of evidence due to systemic under-representation of minority populations; and<br>• Sought input from clinical experts on whether there are distinctive benefits and harms of treatment that may arise for biological, cultural, or social reasons across different communities; and<br>• Confirmed that clinical eligibility criteria have not gone beyond reasonable use of clinical trial inclusion/exclusion criteria to interpret or narrow the FDA label language in a way that disadvantages patients with underlying disabilities unrelated to the condition being treated. | No |
| **For all drugs:** Clinical eligibility criteria that complement the FDA label language may be used to:<br>• Set standards for diagnosis; and/or<br>• Define indeterminate clinical terms in the FDA label (e.g., "moderate-to-severe") with explicit reference to clinical guidelines or other standards; and/or<br>• Triage patients by clinical acuity when the payer explicitly documents that triage is both reasonable and necessary because:<br>○ The size of the population included within the FDA label is extremely large, and there is a reasonable likelihood that many patients would seek treatment in the short term; AND<br>○ The clinical infrastructure is not adequate to treat all patients seeking care and/or broad coverage would create such substantial increases in short-term insurance premiums or other financial strain that patients would be harmed through loss of affordable insurance; AND<br>○ Acuity can be determined on objective clinical grounds and waiting for treatment will not cause significant irremediable harm. | Yes |
| **For drugs with prices or price increases that have been deemed reasonable:** Except for the three purposes outlined above, clinical eligibility criteria should not deviate from the FDA label language in a manner that would narrow coverage. | Yes |
| **For drugs with prices or price increases that have been deemed reasonable:** Documentation that patients meet clinical eligibility criteria should represent a light administrative burden, including acceptance of clinician attestation in lieu of more formal medical record documentation unless documentation is critical to ensure patient safety. | Yes |
| **For drugs with prices or price increases that have been deemed unreasonable:** Clinical eligibility criteria may narrow coverage by applying specific eligibility criteria from the pivotal trials used to generate evidence for FDA approval if implemented with reasonable flexibility and supported by robust appeals procedures as described in the implementation criteria. | Yes |

FDA: U.S. Food and Drug Administration

**Table 6. Step Therapy and Required Switching Fair Design Criteria**

| Step Therapy and Required Switching | |
|---|---|
| **Fair Design Criteria** | **In Scope for this Review?** |
| In order to justify economic step therapy policies extending beyond FDA labeling as appropriate, payers should explicitly affirm or present evidence to document all of the following:<br>• Use of the first-step therapy reduces overall health care spending, not just drug spending | No |
| • The first-step therapy is clinically appropriate for all or nearly all patients and does not pose a greater risk of any significant side effect or harm.<br>• Patients will have a reasonable chance to meet their clinical goals with first-step therapy.<br>• Failure of the first-step drug and the resulting delay in beginning the second-step agent will not lead to long-term harm for patients.<br>• Patients are not required to retry a first-line drug with which they have previously had adverse side effects or an inadequate response at a reasonable dose and duration. | Yes – new threshold of a maximum of 3 steps even if all include appropriate first- line therapies |
| In order to justify required switching policies as appropriate, payers should explicitly affirm or present evidence to document all of the following:<br>• Use of the required drug reduces overall health care spending. | No |
| • The required switch therapy is based on the same mechanism of action or presents a comparable risk and side effect profile to the index therapy.<br>• The required switch therapy has the same route of administration or the difference in route of administration will create no significant negative impact on patients due to clinical or socio-economic factors.<br>• Patients are not required to switch to a drug that they have used before at a reasonable dose and duration with inadequate response and/or significant side effects, including earlier use under a different payer. | No |

FDA: U.S. Food and Drug Administration

**Table 7. Provider Qualifications Fair Design Criteria**

| Provider Qualifications | |
|---|---|
| **Fair Design Criteria** | **In Scope for this Review?** |
| Restrictions of coverage to specialty prescribers are reasonable with one or more of the following justifications:<br>• Accurate diagnosis and prescription require specialist training, with the risk that non-specialist clinicians would prescribe the medication for patients who may suffer harm or be unlikely to benefit.<br>• Determination of the risks and benefits of treatment for individual patients requires specialist training due to potential for serious side effects of therapy.<br>• Dosing, monitoring for side effects, and overall care coordination require specialist training to ensure safe and effective use of the medication. | Yes |
| Requiring that non-specialist clinicians attest they are caring for the patient in consultation with a relevant specialist is a reasonable option when the condition is frequently treated in primary care settings but some elements of dosing, monitoring for side effects, and/or overall coordination of care would benefit from specialist input for many patients. | Yes |

**Table 8. Transparency Fair Design Criteria**

| Transparency | |
|---|---|
| **Fair Access Criteria** | **In scope for this review?** |
| Cost-sharing policies should be presented clearly to consumers prior to health plan selection, allowing all individuals to understand what cost sharing they will face for treatments they are currently taking or are considering. Any significant change to formulary or cost sharing structures should not occur mid-cycle unless plan sponsors include this as a qualifying event allowing plan enrollees to switch plans. | Yes |
| At the point of care, clinicians and patients should be able to rapidly determine the cost-sharing requirements for any treatment along with cost sharing for other alternatives. | No |
| Individuals considering health plan enrollment should be presented with clear information allowing them to understand whether they meet the insurers' clinical criteria for the treatments they are currently taking. The policies should also set out the rationale behind them and be readily understandable. | Yes |
| Clinicians and patients should be able to rapidly determine the clinical criteria for any treatment and view the clinical rationale supporting these criteria. The referenced clinical information should be readily available to the prescribing/ordering provider and the public. | No |
| Individuals considering health plan enrollment should be presented with clear information allowing them to understand whether the treatments they currently take or envision taking will be subject to step therapy or switching policies. | Yes |
| Clinicians, pharmacists, and patients should be able to rapidly determine the requirements related to step therapy and switching policies and be able to easily view a full justification from the insurer. | No |
| Individuals considering health plan enrollment should be able to easily find information related to coverage criteria, including prescriber qualifications, for drugs that they or family members are currently taking. | Yes |
| Clinicians and patients should be able to rapidly determine whether there is a restriction on prescribing for any treatment. Insurers should provide ready assistance to primary care clinicians seeking connection with a relevant specialist for consultation as needed. | No |

*Commentary on Assessment Strategy for Transparency*

For the evaluation of criteria related to prospective plan enrollees' ability to see information about clinical eligibility and cost sharing, we identified the extent to which formulary tiering and clinical eligibility information could be found in publicly available material prior to an individual enrolling in a plan. The goal was to mimic the experience of a prospective enrollee to a new health plan who needed to find out what the requirements for coverage would be and the level of cost sharing for a drug they were already taking. We are aware that payers and plan sponsors may have specialized procedures through which prospective plan enrollees can obtain this information, so we invited each payer to provide information on its approach. We did not evaluate the transparency of this information during the process of care once patients are enrolled in the health plan.

**Table 9. Documentation Burden Fair Design Criteria**

| Documentation Burden | |
|---|---|
| **Fair Access Criteria** | **In scope for this review?** |
| The administrative burden of documenting clinical eligibility should be streamlined and transparent to avoid creating a significant barrier to appropriate care. | Yes |

*Commentary on Assessment Strategy for* Documentation Burden

With prior authorization procedures that are overly burdensome there may be a risk of delayed or abandoned care that could harm patients. In a 2021 Physician Survey conducted by the American Medical Association (AMA) , physicians reported that they and their staffs spend an average of 13 hours each week completing prior authorization forms. It should also always be remembered that prior authorization protocols impose an administrative burden on patients and clinicians that can, by itself, pose a risk to fair access.[5]

To evaluate this element of fair access as part of this 2022 report, we used the material available in the MMIT database and additional information provided by payers to record the number of questions on the prior authorization form for an individual payer and noted any additional documentation requirements that could create additional burden, such as when medical records are explicitly required to be sent to the payer. There is no set threshold for how many questions or additional documentary requirements would qualify as appropriate or not appropriate. Instead, we present findings on the range and variation across payers to provide insight and suggest avenues for determining what level of documentation burden might be accepted as "best practice" under different circumstances.

# 4. Results

Given that we sought to evaluate coverage policies for 19 drug-indication pairs across 18 formularies, there was a maximum of 19 x 18 = 342 possible drug-formulary policy combinations. In each category of fair access, some criteria were not applicable, either because the drug was not covered (but a clinically appropriate alternative was), the drug was not cost-effectively priced (in which case the cost-sharing fair access criteria do not apply), or the drug was considered non-formulary (in which case only cost-sharing criteria can be assessed since payers can be held accountable for the tiering of therapeutic alternatives that are in the formulary). We were able to find all applicable policies between the MMIT database and information provided directly by the payers themselves. MMIT pulls data from a variety of sources known as the MMIT Network, a repository of open-source data including e-prescribing and similar point-of-care solutions, physician educational channels, long-term care and other pharmacies, pharmaceutical manufacturers, and most notably health plans and PBMs. When a policy is not referenced in the MMIT database, it is because MMIT has obtained this information either through a proprietary source, intelligence provided by their network of panelists, and/or other non-publishable digital data assets.

Throughout the report and supplement, numerators and denominators exclude policies for drugs that were determined to be non-formulary, except, as noted, for assessments of the cost-sharing criteria. While this approach does not explicitly penalize a payer in several categories of fair access for excluding a drug from the formulary, it avoids the concern that a payer could receive a favorable rating under clinical eligibility, prescriber restrictions, or step therapy even though the drug is substantially more difficult for a patient to access due to it being non-formulary.

## Concordance by Fair Access Criterion

Our analysis of each individual drug-formulary combination is described in the Supplemental Material. As can be seen in Table 10 below, overall results on concordance with the four fair access criteria domains measured range from a low of 70% for cost-sharing, to a high of 100% for prescriber restrictions.

**Table 10. Number of Coverage Policies Available and Overall Rate of Concordance with Fair Access Criteria Assessed**

| Fair Access Criterion | Drug-Formulary Combinations with Relevant Policies Available out of Applicable Policies, n/N (%) | Concordant Policies, n/N (%) |
|---|---|---|
| Cost sharing | 84/84 (100%) | 59/84 (70%) |
| Clinical eligibility | 322/322 (100%) | 310/322 (96%) |
| Step therapy | 322/322 (100%) | 316/322 (98%) |
| Prescriber restrictions | 322/322 (100%) | 322/322 (100%) |

The percentage of policies judged concordant in Table 10 above uses the number of available policies as the denominator. We believe this is the best single quantitative measure of overall concordance because it does not seem reasonable to reduce concordance rates by including in the denominator policies that are not applicable. However, Table 11 below presents the results with not applicable drug policies as a component of all policies evaluated.

**Table 11. Overall Rate of Concordance with Fair Access Criteria Assessed**

| Fair Access Criterion | Concordant n (%) | Not Concordant n (%) | Not Applicable* n (%) |
|---|---|---|---|
| Cost sharing | 59 (17%) | 25 (7%) | 258 (75%) |
| Clinical eligibility | 310 (91%) | 12 (4%) | 20 (6%) |
| Step therapy | 316 (92%) | 6 (2%) | 20 (6%) |
| Prescriber restrictions | 322 (94%) | 0 (0%) | 20 (6%) |

Note: row totals may not sum to 100% due to rounding.

*Not applicable includes cases when the drug is not covered by the payer. For cost sharing, the criteria are also not applicable for drugs that are not priced within cost-effectiveness levels or that are covered by a payer only through the medical benefit.

### *1. Cost Sharing*

Examples of drugs priced within reasonable cost-effectiveness levels that have a relatively high rate of non-concordance for tier placement included:

- Hemlibra: Seven payers (CVS, OptumRx, Cigna, Anthem, MedImpact**,** Premera, and Florida Blue HIX) do not have this drug on the lowest relevant tier on the pharmacy benefit, and although factor replacement is an alternative treatment option that may be positioned in the preferred brand tier in some formularies, it is not considered an equivalent option in the same class as Hemlibra.

- Nurtec and Ubrelvy: Four payers (Express Scripts, Blue Shield CA, Premera, and Highmark) do not cover any CGRP inhibitors for acute treatment of migraines at the lowest relevant tier.

- Remicade, Renflexis, and Inflectra: Two payers (Anthem, Kaiser HIX) do not cover these or any other targeted immune modulator drugs for the treatment of moderate to severe ulcerative colitis at the lowest relevant tier.

*Exploratory Analysis of Co-Pay / Co-Insurance Amounts*

To gain additional insight into the financial impact of cost sharing on patients, we requested from each payer the most common co-pay/co-insurance amounts selected by plan sponsors for each tier in their formulary. Some high-deductible benefit designs include pharmaceutical spending as part of the deductible, so some patients need to meet that deductible before any coverage with co-pay or co-insurance provisions begins. Because payers offer many different cost-sharing structures, and plan

sponsors can suggest different variations related to the matching of a deductible level with the cost-sharing levels at each tier, the following information should be considered exploratory, and not indicative of a one-size fits all cost-sharing structure for the formulary. For this reason, we are not presenting the information linked to specific formularies.

Of the 17 payers representing the 18 formularies, we received specific cost-sharing amounts from 10 payers representing 10 formularies. Of those 10 formularies, five are three-tier products, four have four or more tiers, and one does not use tiering to determine cost sharing. The most common form of cost sharing in three-tier formularies was based on co-pays for all tiers (4/5), whereas formularies with more than three tiers were most likely to use a mixture of co-pays and co-insurance (3/4).

Table 12 below displays the range of co-pays and co-insurance for each drug across the 10 formularies. The lowest co-pay amount seen was $11 for a 30-day supply, and the highest was $250 for a 30-day supply. The lowest co-insurance rate was 20%, which was the most common rate across all payer formularies except one, for which 50% was the most common. Three payers with a co-insurance structure noted that even with co-insurance, a patient had a maximum dollar amount per 30-day supply ranging from $200 to $350, and the annual out-of-pocket max for the formulary with a 50% co-insurance level was capped at $3,000.

At the drug level, the highest co-pay amount ($250) in any of the formularies was seen for three drugs (Oxbryta, Endari, and Orkambi), and the highest co-insurance amount (50%) was seen in one formulary for ten drugs (Endari, Orkambi, Kalydeco, Symdeko, Trikafta, Humira, Simponi, Xeljanz, Stelara, and Hemlibra).

One three-tier plan had different co-pay amounts within the same tier depending on whether the drug was judged specialty or non-specialty, with a higher co-pay amount required for specialty drugs.

**Table 12. Out-of-Pocket Cost-Sharing Amounts for all Drugs in Scope Across 10 Different Formularies**

| Drug (Brand) | Drug (Generic) | Co-Pay Range (Min-Max)* | | Co-Insurance Range (Min-Max)† | |
|---|---|---|---|---|---|
| Adakveo | Crizanlizumab | $11 | $150 | 20% | 20% |
| Endari | L-glutamine | $11 | $250 | 20% | 50% |
| Entyvio | Vedolizumab | $11 | $150 | 20% | 20% |
| Hemlibra | Emicizumab | $11 | $150 | 20% | 50% |
| Humira | Adalimumab | $11 | $150 | 20% | 50% |
| Inflectra | Infliximab-dyyb | $11 | $150 | 20% | 20% |
| Kalydeco | Ivacaftor | $11 | $150 | 20% | 50% |
| Nurtec | Rimegepant | $11 | $50 | N/A | N/A |
| Orkambi | Lumacaftor/Ivacaftor | $11 | $250 | 20% | 50% |
| Oxbryta | Voxelotor | $11 | $250 | 20% | 25% |
| Remicade | Infliximab | $11 | $100 | 20% | 20% |
| Renflexis | Infliximab-abda | $11 | $150 | 20% | 20% |
| Reyvow | Lasmiditan | $11 | $100 | N/A | N/A |
| Simponi | Golimumab | $11 | $150 | 20% | 50% |
| Stelara | Ustekinumab | $11 | $150 | 20% | 50% |
| Symdeko | Tezacaftor/Ivacaftor | $11 | $150 | 20% | 50% |
| Trikafta | Elexacaftor/tezacaftor/ivacaftor | $11 | $150 | 20% | 50% |
| Ubrelvy | Ubrogepant | $11 | $75 | N/A | N/A |
| Xeljanz | Tofacitinib | $11 | $150 | 20% | 50% |

N/A: these drugs were all subject to a co-pay, and no payers required co-insurance.

*per month supply

† per month supply; many formularies set an out-of-pocket dollar maximum per month (e.g. $200) when co-insurance is applied

### *2. Clinical Eligibility*

There was a very high rate of concordance overall with the fair access criteria related to how payers used the FDA label, clinical trial clinical eligibility criteria, and clinical guidelines to determine the clinical eligibility criteria for coverage. Hemlibra was the only drug for which more than a single formulary did not meet fair access criteria.

- Hemlibra: Four payers (Elixir, Cigna, MedImpact and Florida Blue HIX) in their pharmacy benefit coverage of this drug have restrictions based on severity of disease defined by having a history of bleeding events, a stipulation which is not in the label nor supported by clinical guidelines.

### *3. Step Therapy*

Examples of drugs that have a relatively high rate of non-concordance for the design of step therapy policies include:

- Reyvow: Three payers (United, OptumRx, and BCBS MI) require patients to step through two generic triptans and both Ubrelvy and Nurtec (four steps total) before accessing Reyvow. This exceeds the three-step limit for step therapy, which is independent of whether the drug is deemed to be priced fairly or not.

- Hemlibra: Two payers (United and Florida Blue HIX) require patients with mild or mild to moderate hemophilia to step through a factor replacement product, a step not concordant with fair access criteria given the notably different delivery mechanism of this drug that is preferable to most patients, and the potential desire of younger patients just beginning prophylaxis to avoid factor replacement in order to avoid the development of inhibitors.

Although no single drug had a rate of concordance for step therapy criteria less than 80% across all formularies, we found wide variation in some cases in the number of steps required before receiving coverage for the drug. On the following page, we present in Table 13 the range in the number of steps for each drug across all formularies.

**Table 13. Number of Steps Required for Prior Authorization by Drug**

| Drug (Brand) | Most Common # of Steps | Range | Formularies with Highest Number of Steps |
|---|---|---|---|
| Adakveo | 1 | 0-1 | OptumRx, Premera, MedImpact, Express Scripts, BCBS MI, Blue Shield CA, Cigna, Highmark, United |
| Endari | 0 | 0-2 | United, BCBS MI, Highmark |
| Entyvio | 1 | 0-2 | BCBS MA, MedImpact, OptumRx |
| Hemlibra | 0 | 0-1 | United, Florida Blue HIX |
| Humira | 0 | 0-1 | BCBS MA, Blue Shield CA, CVS, Express Scripts, United, Cigna, HCSC, Florida Blue HIX |
| Inflectra | 1 | 0-2 | BCBS MA, Premera |
| Kalydeco | 0 | 0 | N/A |
| Nurtec | 2 | 0-2 | CVS, United, OptumRx, Anthem, Elixir, Blue Shield CA, BCBS MI, BCBS MA, Premera, Highmark, VHA |
| Orkambi | 0 | 0 | N/A |
| Oxbryta | 0 | 0-2 | United, HCSC, BCBS MI, Highmark, Florida Blue HIX, Elixir |
| Remicade | 1 | 0-2 | Cigna, BCBS MA, OptumRx |
| Renflexis | 1 | 0-2 | BCBS MA |
| Reyvow | 0, 1, 2 | 0-4 | United, OptumRx, BCBS MI |
| Simponi | 1 | 0-4 | BCBS MI |
| Stelara | 1 | 0-1 | BCBS MA, BCBS MI, Blue Shield CA, Cigna, CVS, Elixir, Express Scripts, Florida Blue HIX, HCSC, Highmark, Kaiser, Kaiser HIX, MedImpact, OptumRx, Premera, United, VHA |
| Symdeko | 0 | 0 | N/A |
| Trikafta | 0 | 0 | N/A |
| Ubrelvy | 2 | 0-2 | BCBS MA, BCBS MI, Blue Shield CA, Cigna, CVS, Elixir, Express Scripts, Florida Blue HIX, HCSC, Highmark, MedImpact, OptumRx, Premera, United, VHA, Anthem |
| Xeljanz | 0, 1 | 0-2 | United, BCBS MA, Anthem, Elixir |

BCBS: Blue Cross Blue Shield, CA: California, HCSC: Health Care Service Corporation, HIX: Health Insurance Exchange, MA: Massachusetts, MI: Michigan, N/A: Not applicable due to no step therapy requirement, PBM: Pharmacy Benefit Manager, VHA: Veterans Health Administration

### *4. Provider Restrictions*

Of the applicable policies, 322/322 (100%) were concordant with the fair access criteria for prescriber restrictions.

## Concordance by Drug

Because the drugs included in our analysis can be covered under pharmacy benefits, medical benefits, or both, we had to decide how to report the findings in a way that conveys a fair "apples to apples" comparison across formularies. When a drug was covered by a payer under both the pharmacy benefit and medical benefit, we selected for assessment the coverage policy under the benefit type that was used by the greatest number of payers overall (i.e., the "predominant benefit plan type"). Only pharmacy benefit coverage policies were used to judge cost-sharing concordance for reasons discussed above in Chapter 3. Results for each drug on concordance on all criteria are shown on the following page in Table 14.

Because overall concordance with the fair access criteria was so high, there was not enough variation to explore correlation with features of the drug, drug class, or drug pricing. However, the findings for one drug stand out. Hemlibra for hemophilia A has notably lower rates of concordance for cost sharing and clinical eligibility criteria. This is one of the most expensive drugs in this assessment, and it is used chronically. Hemlibra is also a drug for which there are alternative treatments, albeit treatments that are more expensive on an annual basis for most patients – but not all, depending on their need for regular factor prophylaxis. Therefore, it is not surprising that the utilization management of Hemlibra is more restrictive than for other drugs in this assessment.

**Table 14. Concordance with Fair Access Criteria by Drug: Number (%) of Payers with Concordant Policies out of Payers with Applicable Policies. Concordance Requires Meeting All Applicable Individual Criteria.**

| | Predominant Benefit Plan Type | Cost Sharing | Clinical Eligibility | Step Therapy | Prescriber Restrictions |
|---|---|---|---|---|---|
| **Drug (Indication)** | *(Number of formularies with predominant plan type/number of all formularies)* | *Concordant Policies, n/N* (%)* | *Concordant Policies, n/N* (%)* | *Concordant Policies, n/N* (%)* | *Concordant Policies, n/N* (%)* |
| Adakveo (Sickle Cell Disease) | Medical (13/18) | N/A | 16/16 (100) | 16/16 (100) | 16/16 (100) |
| Endari (Sickle Cell Disease) | Pharmacy (18/18) | N/A | 16/16 (100) | 16/16 (100) | 16/16 (100) |
| Entyvio (Ulcerative Colitis) | Medical (13/18) | N/A | 16/17 (94) | 17/17 (100) | 17/17 (100) |
| Hemlibra (Hemophilia A) | Pharmacy (14/18) | 9/16 (56) | 13/17 (76) | 15/17 (88) | 17/17 (100) |
| Humira (Ulcerative Colitis) | Pharmacy (18/18) | N/A | 17/18 (94) | 18/18 (100) | 18/18 (100) |
| Inflectra (Ulcerative Colitis) | Medical (13/18) | 9/11 (82) | 16/17 (94) | 17/17 (100) | 17/17 (100) |
| Kalydeco (Cystic Fibrosis) | Pharmacy (18/18) | N/A | 17/17 (100) | 17/17 (100) | 17/17 (100) |
| Nurtec (Migraine: Acute Therapies) | Pharmacy (18/18) | 12/18 (67) | 18/18 (100) | 18/18 (100) | 18/18 (100) |
| Orkambi (Cystic Fibrosis) | Pharmacy (18/18) | N/A | 17/17 (100) | 17/17 (100) | 17/17 (100) |
| Oxbryta (Sickle Cell Disease) | Pharmacy (15/18) | N/A | 15/15 (100) | 15/15 (100) | 15/15 (100) |
| Remicade (Ulcerative Colitis) | Medical (13/18) | 10/12 (83) | 16/17 (94) | 17/17 (100) | 17/17 (100) |
| Renflexis (Ulcerative Colitis) | Medical (13/18) | 7/9 (78) | 15/16 (94) | 16/16 (100) | 16/16 (100) |
| Reyvow (Migraine: Acute Therapies) | Pharmacy (16/18) | N/A | 15/15 (100) | 12/15 (80) | 15/15 (100) |
| Simponi (Ulcerative Colitis) | Pharmacy (16/18) | N/A | 17/18 (94) | 17/18 (94) | 18/18 (100) |
| Stelara (Ulcerative Colitis) | Pharmacy (17/18) | N/A | 17/18 (94) | 18/18 (100) | 18/18 (100) |
| Symdeko (Cystic Fibrosis) | Pharmacy (18/18) | N/A | 17/17 (100) | 17/17 (100) | 17/17 (100) |
| Trikafta (Cystic Fibrosis) | Pharmacy (18/18) | N/A | 18/18 (100) | 18/18 (100) | 18/18 (100) |
| Ubrelvy (Migraine: Acute Therapies) | Pharmacy (18/18) | 12/18 (67) | 17/17 (100) | 17/17 (100) | 17/17 (100) |
| Xeljanz (Ulcerative Colitis) | Pharmacy (18/18) | N/A | 17/18 (94) | 18/18 (100) | 18/18 (100) |

N/A: Not applicable (meaning that these drugs are not priced at a cost-effective level (n=13) and therefore the cost sharing criteria do not apply)

*The total N for each fair access criteria represents whether the specific criterion is applicable for that drug.

## Concordance by Formulary

As shown in Table 15 on the following page, the percent concordance across all 19 drugs on specific fair access criteria varies widely between formularies, ranging from 0% to 100% for cost sharing, 56% to 100% for clinical eligibility, 89% to 100% for step therapy, and 100% for all payers for prescriber restrictions. One limitation in interpreting the specific findings for individual formularies should be emphasized: the relatively small number of drug policies applicable for assessment, particularly in the cost sharing domain. The small number of relevant policies in this domain, ranging from two to six, means that the difference in the rating for a single drug leads to very large absolute differences in the percentage of concordance with fair access criteria. Therefore, we advise readers of these results to avoid making strong interpretations of relative performance across formularies in meeting cost sharing criteria.

It should also be noted that not all formularies could be assessed on all criteria for the full set of 19 drugs. In Table 15, for each payer, the total 'N' between criteria differs across payers when some payers covered particular drugs only on the medical benefit, or when drugs were excluded from the formulary. As mentioned, the cost-sharing criteria are only applicable if the drug is priced at a cost-effective level and is covered by the payer under a pharmacy benefit. For non-formulary drugs, cost-sharing criteria are applicable since they can be applied to the formulary placement of reasonable alternatives in the same drug class, but the remaining criteria would not apply.

**Table 15. Rate of Concordance by Individual Payer: Number (%) of Policies Meeting Each Fair Access Criterion out of all Relevant Policies**

| | Cost Sharing | Clinical Eligibility | Step Therapy | Prescriber Restrictions |
|---|---|---|---|---|
| **Payer/PBM (Largest Formulary)** | *Concordant Policies*, n/N† (%)* | *Concordant Policies, n/N (%)* | *Concordant Policies, n/N (%)* | *Concordant Policies, n/N (%)* |
| **Three-Tier Formularies** | | | | |
| CVS | 3/4 (75) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| Express Scripts | 3/5 (60) | 17/17 (100) | 17/17 (100) | 17/17 (100) |
| United | 3/3 (100) | 19/19 (100) | 17/19 (89) | 19/19 (100) |
| Cigna | 4/5 (80) | 15/16 (94) | 16/16 (100) | 16/16 (100) |
| OptumRx | 5/6 (83) | 18/18 (100) | 17/18 (95) | 18/18 (100) |
| BCBS MA | 5/5 (100) | 10/18 (56) | 18/18 (100) | 18/18 (100) |
| MedImpact | 5/6 (83) | 18/19 (95) | 19/19 (100) | 19/19 (100) |
| Highmark | 1/3 (33) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| Premera | 3/6 (50) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| BCBS MI | 3/3 (100) | 19/19 (100) | 17/19 (89) | 19/19 (100) |
| Elixir | 6/6 (100) | 13/14 (93) | 14/14 (100) | 14/14 (100) |
| **Four-Tier Formularies** | | | | |
| Anthem | 2/6 (33) | 10/10 (100) | 10/10 (100) | 10/10 (100) |
| Blue Shield CA | 0/2 (0) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| Kaiser HIX | 1/6 (17) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| **Other** | | | | |
| VHA | 6/6 (100) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| Kaiser | 4/6 (67) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| HCSC | 3/3 (100) | 19/19 (100) | 19/19 (100) | 19/19 (100) |
| Florida Blue HIX | 2/3 (67) | 18/19 (95) | 18/19 (95) | 19/19 (100) |

BCBS: Blue Cross Blue Shield, CA: California, HCSC: Health Care Service Corporation, HIX: Health Insurance Exchange, MA: Massachusetts, MI: Michigan, PBM: Pharmacy Benefit Manager, VHA: Veterans Health Administration

* Six drugs were priced at a cost-effective level and therefore cost-sharing criteria apply to only these six drugs.

† N for the cost sharing criteria represents only those policies (out of six) that are applicable for assessment. N for the remaining three criteria may not always total 19 due to criteria being not applicable for some drugs. For example, if a drug is not covered (which occurred in seven drug/formulary combinations) the criteria would not be applicable for those drugs. Criteria may also not be applicable if the drug is considered non-formulary. In these instances, cost-sharing criteria may be applicable if the drug is priced at a cost-effective level and is covered on a pharmacy benefit plan; the remaining criteria would not apply.

## Transparency and Prior Authorization Burden Summary

In addition to core analyses of concordance with fair access criteria for cost sharing, clinical eligibility criteria, step therapy, and prescriber restrictions, we evaluated concordance on a select set of drugs and formularies on criteria related to the transparency of cost sharing and clinical eligibility criteria to prospective plan enrollees, and the relative burden of the questions needed to be answered to obtain coverage as part of prior authorization.

### *Transparency*

For the purposes of this analysis, we evaluated select migraine (Nurtec, Ubrelvy) and ulcerative colitis treatments (Humira, Xeljanz). We knew that these drugs would be subject to relatively high levels of prior authorization and that payers were likely to use tiering as part of their approach to negotiating prices with manufacturers. To evaluate the transparency criteria described in Table 8 earlier in this report, we simulated the experience of individuals shopping for health plans who would want to understand whether they would meet the clinical criteria of a prospective new payer for the drug of their choice, and what the level of cost sharing would be for that drug. For the selected drugs, we therefore conducted an internet search for each payer's covered drug lists and determined whether the information available was adequate to inform a prospective new enrollee. The results of this exploratory assessment of transparency are shown in Table 16 on the following page.

**Table 16. Concordance with Transparency Criteria**

| Payer | Formulary | Payer/PBM | Tier Information | Clinical Eligibility Details |
|---|---|---|---|---|
| CVS | CVS Caremark Standard w/ Advanced Specialty Control | PBM | N | N |
| Express Scripts | Express Scripts National Preferred with Advantage Plus | PBM | N | N |
| United | UnitedHealthcare Advantage Three Tier | Payer | Y | Y |
| VHA | VHA National Formulary | Payer | Y | Y |
| Cigna | Cigna Standard Three Tier | Payer | Y | Y |
| OptumRx | OptumRx Select Standard | PBM | Y | N |
| Kaiser | Kaiser Permanente Southern California | Payer | Y | N |
| Anthem | Anthem Essential 4 Tier | Payer | Y | Y |
| BCBS MA | BCBS Massachusetts Three Tier | Payer | Y | Y |
| HCSC | BCBS of Illinois Basic 6 Tier | Payer | Y | N |
| Elixir | Elixir Standard Formulary | PBM | Y* | Y |
| MedImpact | MedImpact Portfolio High Formulary | PBM | Y* | N |
| Highmark | Highmark Blue Cross Blue Shield 3 Tier | Payer | Y | Y |
| Blue Shield CA | Blue Shield of California Plus Formulary | Payer | Y | N |
| Florida Blue HIX | Florida Blue Care Choices HIX | Payer | Y | Y |
| Premera | Premera Preferred 3-Tier – B3 | Payer | Y | Y |
| Kaiser HIX | Kaiser Permanente California HIX | Payer | Y | N |
| BCBS MI | BCBS of Michigan Custom 3 Tier | Payer | Y | Y |

Y: Transparent; full coverage details are publicly available, N: Not transparent; there is no information or full details are missing

* Explicit out-of-pocket cost sharing information is available

The majority (16/18) of payers had updated prescription drug lists available on the internet for their respective formularies, complete with tier placement and notice of any restrictions in place for utilization management (e.g., prior authorization, step therapy, or quantity limits). Two payers, Elixir and MedImpact, explicitly described out-of-pocket costs patients could expect for their treatments depending on tier placement, including exact co-pay dollar amounts or percentages for co-insurance per monthly supply. CVS and Express Scripts were the only two payers for which we could not find adequate cost sharing information in the public domain. CVS provided details on covered medications, co-pay/co-insurance, and formulary alternatives via a patient portal for enrolled members, but not to the public. Express Scripts provided no direct access to information on its National Preferred Formulary. Its drug lists were available online via one of its payer clients, but since this information did not come directly from the controlling PBM and may not reflect their standard coverage policy, we judged this public information inadequate to meet our transparency criterion.

Payers provided relatively less transparency into prior authorization policies than tiering, with only 9/13 (64%) of health insurers providing detailed criteria outlining requirements for clinical eligibility, step therapy, and provider restriction. We judged that four payers (Kaiser, HCSC, Blue Shield CA, Kaiser HIX) did not provide sufficiently detailed coverage criteria, such as age cutoffs, diagnosis criteria, symptom frequency/severity, etc.

Most PBMs included in this assessment (4/5=80%) do not provide publicly available coverage policies under their own name. This is likely a reflection of the PBM business model, in which they provide services through a health plan that is the primary portal through which enrollees (or potential enrollees) could learn about their coverage.

***Prior Authorization Burden***

To understand the level of burden on clinicians seeking coverage authorization to initiate a new prescription, we assessed actual prior authorization forms to determine how many questions they included and whether additional medical documentation was required. Prior authorization forms were obtained from the MMIT database as well as directly from six payers. We selected to evaluate these forms for a sample of 11 drugs because these drugs were in competitive drug classes in which we believed more variation would be found in prior authorization burden. The 11 drugs were Nurtec, Reyvow, and Ubrelvy for acute migraine; and Humira, Simponi, Remicade, Renflexis, Inflectra, Xeljanz, Stelara, and Entyvio for ulcerative colitis.

We developed a standardized method of counting the number of questions physicians would be required to answer to initiate a patient on one of the 11 included drugs. Results are shown in Table 17 on the following page. We assigned a value of 1 to each question type identified in the evaluated prior authorization forms (yes/no, open-ended, multiple-choice question, open field, etc.). To assess the median and range of required questions for each drug, questions were separated into two categories, "general" and "patient-specific":

- General questions are questions the clinician would be required to answer for *any* patient to initiate a new therapy (e.g., patient name, date of birth, etc.). The median number of required questions reported in Table 17 is based on these general questions.
- Patient-specific questions are questions that depend on a patient's medical history and clinical eligibility status (e.g., "If a patient has previously tried X therapy, did they respond?" Y/N). To account for the patient-to-patient variation in documentation burden, we present a range of the possible combined number of required 'general' and 'patient-specific' questions in Table 17.

**Table 17. Documentation Burden**

| Drug | Median Number of Required Questions (Range) | Medical Documentation Required by at least one payer? (Y/N) |
|---|---|---|
| **Acute Migraine** | | |
| Nurtec | 34 (27-56) | N |
| Reyvow | 36 (26-51) | N |
| Ubrelvy | 34 (25-56) | N |
| **Ulcerative Colitis** | | |
| Entyvio | 27 (26-51) | Y |
| Humira | 25 (22-51) | Y |
| Inflectra | 28 (22-47) | Y |
| Remicade | 28 (24-47) | Y |
| Renflexis | 33 (22-47) | N |
| Simponi | 31 (25-63) | Y |
| Stelara | 28 (25-67) | Y |
| Xeljanz | 30 (26-71) | Y |

N: no, Y: yes

All prior authorization forms evaluated in this analysis included language recommending submission of supporting documentation (i.e., documentation of diagnosis, previous treatments, or previous medications). At least one payer required formal medical documentation for all of the ulcerative colitis drugs except Renflexis.

## Changes to Payer Coverage Policies After Draft Analysis

Draft results of this analysis were shared with all payers on July 28, 2022. Payers were given three weeks to submit comments and were invited to provide corrections, updates, and perspectives that might justify any policy not meeting fair access criteria. As part of the feedback received from payers, five payers informed us that they were in the process of changing coverage policies on a total of 11 drugs in ways that would move coverage into concordance with the fair access criteria. Most of these changes affected tier placement, however some reflected changes to clinical eligibility criteria. In order to preserve the integrity of the analysis, we have not included these changes in the primary results presented above. But to capture the status of these policies as of the time of the publication of this report, and to suggest how coverage policies may evolve to meet fair access criteria, we summarize these changes in Table 18 below and calculate the hypothetical updated concordance rate based on these changes.

**Table 18. Changes to Payer Policies After July 28, 2022 That Achieved Concordance with Fair Access Criteria**

| Payer | Drug | Policy Change | Concordance with Policy Change Included |
|---|---|---|---|
| Anthem | Oxbryta | Effective August 1, 2022, updated clinical eligibility criteria for Oxbryta to include patients ages 4 and older. | Clinical Criteria 10/10 (100%) |
| BCBS MA | Entyvio | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Inflectra | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Humira | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Remicade | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Renflexis | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Simponi | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Stelara | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| | Xeljanz | Effective August 31, 2022, preferred agents now require a trial of one conventional therapy; non-preferred agents require a trial of one conventional therapy and one preferred biologic. | Clinical Criteria 18/18 (100%) |
| Blue Shield CA | Revyow | Effective January 1, 2023, will move at least one CGRP agent to the preferred brand tier. | Cost Sharing 2/2 (100%) |
| MedImpact | Hemlibra | Effective December 1, 2022, the requirement for at least 2 bleeding episodes per year in patients with mild or moderate hemophilia A without inhibitors was removed. | Clinical Criteria 19/19 (100%) |
| | Hemlibra | Effective January 1, 2023, Hemlibra will be moved to a preferred brand tier. | Cost Sharing 6/6 (100%) |
| Premera | Hemlibra | Effective October 1, 2022, Hemlibra was moved to preferred brand tier. | Cost Sharing 5/6 (83%) |

BCBS: Blue Cross Blue Shield, CA: California, MA: Massachusetts

# 5. Input from Patient Groups

As part of our 2022 assessment of Barriers to Fair Access, we reached out to the following disease-specific patient organizations across the five therapeutic areas represented by the drugs in this year's report to enhance understanding of real-world patient experience with access:

- Coalition for Headache and Migraine Patients (CHAMP)
- Crohn's and Colitis Foundation
- Crohn's and Colitis Young Adults Network
- Cystic Fibrosis Foundation
- Headache and Migraine Policy Forum
- Hemophilia Federation of America
- National Hemophilia Foundation
- Sick Cells
- Sickle Cell Association of the Midwest

Several of the patient organizations we spoke with have priority initiatives to tackle access challenges faced by their communities and have thus documented through surveys, patient stories, and patient access case work the significant financial and health impacts of not having appropriate and timely access to care.

For example, the 2019 Cystic Fibrosis Foundation Health Insurance Survey found that 1,011 (75%) of 1,341 people with cystic fibrosis report their insurance limits the type of prescription medications they can access, and 504/1,854 (27%) reported that the availability or quality of health insurance has affected their decision about which state to live in.[6] In a survey of 1,770 migraine patients performed by CHAMP, 1,682 (95%) of respondents reported that they have experienced at least one access barrier, and 1,328 (75%) say they have experienced more than three challenges to getting their needed medicines.[7] The Crohn's & Colitis Foundation's 2018 Advocacy and Access to Care survey found that 259/1015 (25%) of survey respondents needed to appeal an adverse health insurer decision resulting in delayed access to treatment. This finding was echoed by the stories ICER staff heard directly from leaders of the Crohn's and Colitis Young Adult Network, who told us about the negative impact of such delays on their health outcomes as well as life and educational goals.

Patient organizations also provided stories that illustrate the patient experience of cost sharing, step therapy, and restrictions on prescribers. These are provided below to serve as a reminder of the realities faced by people across our health system in trying to access appropriate care, but it is important to note that these examples are not necessarily the result of any payer policies evaluated in this report.

***Cost Sharing***

While prescription medications are just one of many different costs related to health care, cost-sharing provisions are a significant contributor to the financial toxicity faced by many patients and their families. Beyond the cost-sharing criterion that we were able to evaluate in this report, the broader set of ICER fair access criteria specify that cost sharing should not be structured primarily to shift health care costs to patients when they have few or no lower cost options that are medically appropriate. Yet, the patient communities involved in this year's report have highlighted several examples of the increasing financial pressure due to cost-sharing policies.

For people living with cystic fibrosis, 821 (44%) of 1,856 of respondents to the aforementioned Health Insurance Survey reported having challenges paying for at least one medication or service associated with their treatment regimen in the past year, and 172 (15%) of 1,165 of people reported having problems affording their modulator therapy – even before Trikafta was introduced.[6,8]

The advent of co-pay accumulators and maximizers has further exacerbated affordability challenges for people living with cystic fibrosis and other conditions, especially when there are no lower cost or alternate treatment options. One person with cystic fibrosis reported a high out of pocket responsibility of more than $11,000 per month. Manufacturer co-pay assistance was limited to $8,900 per month, which left this person with an out-of-pocket responsibility of over $2,100 per month. With such a high out-of-pocket requirement each month, normally this person would reach their out-of-pocket maximum for the year in the first or second month (depending on whether they had an individual or a family plan). Because the payer used a co-pay accumulator, the manufacturer assistance did not count toward this person's out-of-pocket maximum, so they would not hit that maximum until month five at the earliest (Cystic Fibrosis Foundation, email communication, June 2022). At times, even a small cost sharing amount can be the difference between access and no access, especially for people navigating many different costs associated with their disease.

For people affected by migraine, high out-of-pocket and co-pays have led some patients to seek treatment at the emergency room or urgent care since they could not afford the medications their doctors prescribed for them. One patient in her mid-20s described a series of access challenges:

> "I was diagnosed with chronic intractable migraine when I was 25, but when I turned 27 four years ago and lost access to my mom's amazing insurance with out-of-network benefits and low deductibles, I have spent a significant amount of money a year managing my migraine disease and associated comorbidities, and the past two years spent more on medical care than I made working. I have also experienced step therapies and large cost sharing due to my specific insurance, and it is challenging every time I have tried to go back

to work when my COBRA expires. However, I still try to go back to work/school. I always fear my treatment plan will be disrupted." (CHAMP, email communication, June 2022)

Several patient groups also noted that because many of the conditions they live with are serious and require several chronic medications and other costly interventions, financial toxicity can quickly accumulate, which can lead to delays in care or other access challenges.

### *Clinical Eligibility Criteria*

We received numerous examples from patient organizations of onerous clinical eligibility documentation requirements faced by patients and their care teams in trying to navigate insurance for their medications. The following anecdotal examples are reports from individual patients and are not generalizable to all patients in every condition covered in this report.

For a patient with cystic fibrosis covered by a self-funded employer plan, a request for Trikafta was denied because the insurer said the patient did not have cystic fibrosis. The patient's care team appealed, sent two forms of documentation proving the existence of an eligible mutation and yet were still denied. The second appeal was supported by Compass, the Cystic Fibrosis Foundation's personalized support service for insurance, legal, and financial challenges that was ultimately able to confirm the medication approval through a specialty pharmacy (Cystic Fibrosis Foundation Compass, email communication, June 2022).

A different patient with cystic fibrosis was denied Trikafta on the basis of not having approved mutations and, due to the slow internal process to update coverage criteria upon Trikafta's approval, the insurer would not approve access despite proof of the mutation (Cystic Fibrosis Foundation, email communication, June 2022).

The ICER fair access clinical eligibility criteria stipulate that even for drugs with prices or price increases that have been formally deemed unreasonable, which would include Trikafta, neither the documentation requirements nor administrative burden should result in major barriers to care for patients who meet criteria, and payers should publicly post annual evaluations for each drug of rates of ultimate coverage approval following initial coverage denial due to documentation failures.

### *Step Therapy*

The patient communities we spoke with have many examples documenting the emotional frustration and health impact of step therapy protocols. This was evident in the patient stories shared by the Crohn's and Colitis Foundation.

One patient, who had been diagnosed at age 15 and effectively treated with a biologic medicine, described her journey after her parents switched insurance providers to one which no longer covered the medication. The new step therapy protocol she was required to go through led to a

worsening of her symptoms and accumulation of antibodies so that her original medication was no longer effective when she was finally able to access it again.[9]

Another Crohn's patient had lived with controlled symptoms for more than two decades when his insurance suddenly required him to try another drug before they would resume coverage for the drug that had kept him in remission for so long. It took nearly three months to bring his symptoms back under control once he was allowed to return to the original medication.[10]

***Other Significant Patient Experience***

While our analysis is limited to a subset of the overall ethical framework for Fair Access, it is important to highlight significant barriers that we were not able to systematically evaluate.

One of those is the impact of social determinants of health and systemic racism in the health care system that has resulted in many people with sickle cell disease not being able to access appropriate care. Members of the sickle cell patient community informed us that lack of trust in the health care system and current treatments mean patients are not having the opportunity to potentially benefit from advancements in treatment. A new report by the sickle cell patient community "Medicaid Access & Landscape Review: Opportunities to Improve Access" provides a comprehensive view of such barriers to access.[11]

These issues are also evident for patients with severely disabling chronic migraine who said:

> "Being a frequent flyer to the ER, they now know me well. Decades ago, I was discriminated against for having migraine disease. Called a drug seeker. But through advocacy for myself and others I'm fortunate enough to get the treatment needed and with empathy."[12]

Another patient described the experience of feeling judged by the specialist:

> "One of the biggest challenges I faced was being seen holistically. Once I was judged by the only migraine specialist in my network. He wrote in my chart that my pain was psychological 'there's no way I could be in pain every day' and that I'd be better seen at behavioral health. Once he wrote those things no one cared to help me. I felt blacklisted. But that did not stop me from making complaints. Ever since then I have vowed to make strides every day to advocate for myself and people like me that live with invisible disabilities." (CHAMP, email communication, June 2022)

As these data and patient stories illustrate, many patients face access challenges that do not align with the ethical framework of providing patients with fair access to medicines. In a system notable for high drug prices and tight utilization management, patients with serious conditions often lose. These stories are meant to highlight what is difficult to evaluate through our focus on insurance coverage policy documents.

# 6. Discussion

This assessment set out to evaluate whether coverage policies for drugs evaluated in ICER reviews during the calendar year 2020 were covered by major payers as of July 14, 2022 in concordance with fair access criteria for cost sharing, clinical eligibility criteria, step therapy, and prescriber restrictions. As noted in the introduction, this assessment was not able to evaluate many critical elements of how these coverage policies are administered in the real world, including how transparent and efficient the prior authorization process is to clinicians and patients, and how responsive payers are to requests for medically appropriate exceptions. These limitations are important in framing the results of the assessment, which found a high level of concordance of coverage policies with fair access criteria across the formularies with the highest number of covered lives of large private payers in the United States.

Despite the high overall level of concordance, failure to meet cost sharing, clinical eligibility, and step therapy criteria was clustered in a few drugs or condition areas. Foremost among these was the drug Hemlibra for hemophilia. As noted earlier, seven payers did not have Hemlibra on the lowest relevant tier despite its price being cost-effective in comparison to factor replacement therapy. In addition, four payers have coverage restrictions based on severity of disease defined by having a history of bleeding events, a stipulation which is not in the label nor supported by clinical guidelines; and two payers require patients with mild or mild to moderate hemophilia to step through a factor replacement product, a step not concordant with fair access criteria given the notably different delivery mechanism of this drug that is preferable to most patients, and the potential desire of younger patients just beginning prophylaxis to avoid factor replacement in order to avoid the development of inhibitors. In some cases, the failure of coverage to meet fair access criteria may arise from some payer's desire to restrict access for patients with "mild" hemophilia – those patients who have never used factor prophylaxis before. This is not unreasonable, but young patients who are just entering an age and/or activity range that make them suitable candidates for prophylaxis may want to start with Hemlibra instead of factor in order to eliminate the risk of developing inhibitors, and therefore we have judged coverage language that does not provide an easy pathway for initiating prophylaxis with Hemlibra to be inconsistent with fair access criteria.

Certain drugs in two other treatment areas were also more likely to have coverage that did not meet fair access criteria. Nurtec and Ubrelvy for acute migraine, Remicade, Renflexis, and Inflectra for ulcerative colitis, are all drugs judged to be priced within reasonable cost-effectiveness levels. However, multiple payers did not assign at least one drug or relevant comparator to the lowest relevant tier. An excessive number of steps in step therapy also led to lower concordance with fair access criteria for the migraine drugs. The combination of required steps through different triptans and preferred CGRP options resulted in more than three total steps for some patients, a number of steps that we had decided to use as a maximum threshold for the analysis this year.

Whereas it may seem reasonable to expect competitive classes such as acute migraine drugs to see heavier use of prior authorization and step therapy to shift utilization to preferred options, the coverage restrictions for Hemlibra, which faces no competition from a true therapeutic alternative, are likely driven by its high cost and the resulting attempt to target use only toward more severely affected patients with clinical "phenotypes" that clearly demonstrate a need for regular prophylaxis.

The results of this assessment suggest that for the subset of fair access criteria we could evaluate, most coverage policies across these formularies are structured to provide fair access for this set of drugs. Payers have a responsibility to use evidence to establish prudent limits to coverage, and when structured appropriately and administered well, these policy tools can in many cases be important in protecting patients from the risks of care outside of established evidentiary boundaries. Moreover, it is important to recognize the financial stewardship that is delegated to payers in the US. Spending on health care is anticipated to continue to grow faster than the overall economy, leading to pressure on state and federal budgets as well as on the ability for employers and private payers to maintain affordable health insurance.[13] Increased spending on drugs is an important contributor to overall health care spending, lending ethical justification to the efforts by payers to use policies such as step therapy to negotiate lower drug prices in ways that will not adversely affect patient outcomes.[14,15] In the main, our results suggest that the payers in this assessment are meeting that challenge in a way that conforms with the broad outlines of criteria for fair access.

However, as noted throughout this report, there are important limitations to our analysis which should color any conclusions. Perhaps foremost, we were unable to evaluate many of the aspects of coverage policy implementation, such as the ease of obtaining medical exceptions, that are at the heart of many of the barriers experienced by clinicians and patients to appropriate coverage. It is also possible that the 18 formularies selected for this assessment provide coverage more consistent with fair access criteria than formularies covering fewer individuals offered by the same payers, or than formularies from smaller payers.

Indeed, the results of this report do not negate findings from other work documenting the barriers that coverage policies can present to appropriate, timely care. For example, a 2018 Physician Survey conducted by the American Medical Association on prior authorization found that 65% of providers had to wait, on average, at least one business day within the previous week before receiving a prior authorization decision from a health plan, and 26% of providers waited three business days or more.[16] A recent compilation and analysis of the existing peer-reviewed and professional literature estimated that payers, manufacturers, physicians, and patients together incur approximately $93.3 billion in costs annually on implementing, contesting, and navigating utilization management.[17]

Our exploratory analyses addressed some elements of these concerns. Within the set of drug coverage policies assessed, the "simplest" prior authorization form among the payers whose forms were available for review required answers to 22 separate questions. Factoring in variations in patient characteristics, many prior authorization forms would have required clinicians to answer somewhere between 40-50 questions, the highest range extending up to 71 separate questions. Even if many of these questions are relatively straightforward, the time to fill out these forms and the risk that some elements will be missed or entered in error seems high, even if these forms are administered through electronic formats.

Our other exploratory analysis in this report looked at whether prospective health plan enrollees would have been able to use internet searches to find adequate information on whether formularies would cover their currently prescribed medication, and, if so, at what cost sharing level. Although information on cost sharing was widely available, only 64% of the payers had adequately accessible information detailing the clinical eligibility criteria under which coverage would be approved for specific drugs. Improving the availability of this information for people who are considering switching insurers, or who must switch due to a change in employment, should be an important goal for payers seeking to meet the broad goals of fair access.

The concerns regarding barriers to fair access are equal if not greater for policies requiring step therapy that go beyond the "clinical" steps included in the FDA label. Although step therapy is justifiable when used to encourage use of safer, better established treatment options, or the use of equally effective therapies at lower cost, some studies have demonstrated negative effects on patient outcomes related to step therapy, and there has been a consistent push from many patient groups, clinical societies, and commentators to add meaningful consumer protections and transparency to step therapy policies not rooted in the FDA label.[18] Our results suggest that the vast majority of payers in this study have step therapy coverage policies designed to meet key fair access criteria, but we are unable to assess whether the implementation of these policies matches the high standards needed to avoid potentially harmful consequences for patients.

Finally, as with our prior report, one important "result" of this assessment was a series of changes to payer policies that brought coverage into concordance with fair access criteria. Payers informed us after receiving draft assessment results of changes to coverage for 11 drugs. In some cases, these changes were minor clarifications of clinical eligibility criteria, but other policy changes included more substantial broadening of coverage or important shifts in tiering placement that would lead to lower out-of-pocket cost sharing for patients. Insurers and PBMs may continue to reflect further on their own procedures and approaches to coverage determination, and we encourage patient advocates and clinicians to continue to engage on these issues. We believe that the changes made during this assessment suggest an openness and an opportunity to use fair access criteria to achieve a more consistent approach to providing fair access for drugs.

# 7. Conclusion

This assessment has been presented as much as a sign of the limitations in the evidence available to us – and to the public – as it has been a report that can give important insights into the current status of insurance coverage for drugs in the US. As such, it is likely to fully satisfy no one. It will leave some patient advocates and clinician representatives feeling a disconnect between the overall high marks given to payer formularies and their lived experience with cost sharing and prior authorization. Conversely, payers may feel that too much emphasis has been given to the minority of examples in which coverage policies were judged not to meet fair access criteria and, in particular, that there are contextual factors behind tiering decisions and the actual amounts that patients pay out of pocket that render our judgments superficial and potentially misleading.

All are right to some extent. One important conclusion from this assessment is that there should be greater transparency regarding how insurers frame and implement their coverage policies. Transparency certainly for affected patients and their clinicians, but also for the broader research community and the public. Coverage policies and tiering have been treated by some companies as competitive assets, held in confidence, and used to seek advantages against rivals. Other payers post all their policies publicly. Only with greater transparency across the entire industry will payers be able to demonstrate fully their commitment to the appropriate application of evidence to insurance coverage. And only with greater transparency will payers' call for fair pricing be heard by the public with the power it deserves.

However, despite the limitations we have highlighted, we hope this report stimulates further action. Payers should be accorded credit where credit is due: the evidence available and the limitations of our research effort leave many questions, but the great majority of payer policies in the formularies evaluated are structured in a way to support many key elements of fair access. In addition, the changes in coverage policies noted following initial assessment mirror those seen during the process of the first report in 2021 and show that payers are listening, and that transparency may lead to positive change. This assessment was never meant to produce a definitive evaluation of fair access for pharmaceuticals. We hope that it helps move all participants in the health system toward greater understanding and dialogue. In closing, we wish to note again that underlying this effort is the white paper on Cornerstones for Fair Access that was produced with substantial guidance and input from members of the ICER Policy Leadership Forum. We wish to acknowledge and thank the participants in that effort, and those individuals who gave us continued input as part of our Working Group for this assessment. None of these individuals, or organizations, should be viewed as agreeing with this assessment, and any errors in this paper are solely the responsibility of the authors. To all, however, we give our thanks and our praise for their honesty and willingness to pursue a common goal from different starting points.

# References


1. Lenahan KL, Nichols DE, Gertler RM, Chambers JD. Variation In Use And Content Of Prescription Drug Step Therapy Protocols, Within And Across Health Plans. *Health Affairs.* 2021;40(11):1749-1757.
2. Kaiser Family Foundation, Claxton G, Rae M, Young G, McDermott D, Whitmore H, Kerns J, Cifuentes J, Damico A, Strange L. *Employer Health Benefits 2020 Annual Survey.* United States of America October 08, 2020.
3. Ornstein C. Are Copay Coupons Actually Making Drugs More Expensive? ProPublica. https://www.propublica.org/article/are-copay-coupons-actually-making-drugs-more-expensive. Published 2016. Accessed September 21, 2022.
4. Fein A. Copay Accumulator update; Widespread Adoption as Manufacturers and Maximizers Limit Patient Impact. Drug Channels. https://www.drugchannels.net/2018/09/copay-accumulator-update-widespread.html. Published 2018. Accessed September 21, 2022.
5. American Medical Association. 2021 AMA prior authrorization (PA) physician survey American Medical Association https://www.ama-assn.org/system/files/prior-authorization-survey.pdf. Published 2022. Accessed January 6, 2023 2023.
6. George Washington University. Cystic Fibrosis 2019 Health Insurance Survey. In:2019.
7. Coalition for Headache and Migraine Patients. Understanding the Patient Experience: CHAMP Headache Disease Patient Access Surveys, Brief 1-3. https://headachemigraine.org/headache-study-brief-1-and-2/. Accessed July 28, 2022.
8. Seyoum S, Regenstein M, Nolan L. Cost, coverage, and the underuse of medications among people with CF. *Health Policy and Management Issue Briefs.* 2020;Paper 57.
9. Crohn's and Colitis Foundation. Advocacy Story: Sydney's Story. https://www.crohnscolitisfoundation.org/personal-stories/sydney. Accessed July 26, 2022.
10. Crohn's and Colitis Foundation. Advocacy Story: Robert's Story. https://www.crohnscolitisfoundation.org/personal-stories/robert. Accessed July 26, 2022.
11. Sick Cells and Avalere Health. *Medicaid Landscape and Access Review for Prescription Drugs Treating Sickle Cell Disease.* 2022.
12. Migraine Meanderings, The Headache and Migraine Policy Forum. Survey Report: Migraine in the ER and Urgent Care. https://www.migrainemeanderings.com/empowering-patient-voices/patient-surveys. Published 2022. Accessed July 26, 2022.
13. Peter G. Peterson Foundation. Healthcare Costs For Americans Projected to Grow at an Alarmingly High Rate In. Vol 2023: Peter G. Peterson Foundation; 2019.
14. Sherman MC, Gregory D; Parent, Jason; Katarina Wagner, Anita Prescription Medications Account For One In Four Dollars Spent By A Commercial Health Plan. In. Vol 2023: Health Affairs Blog; 2018.
15. Rome BN, Egilman AC, Kesselheim AS. Trends in Prescription Drug Launch Prices, 2008-2021. *Jama.* 2022;327(21):2145-2147.
16. American Medical Association. 2018 AMA prior authorization (PA) physician survey. https://www.ama-assn.org/system/files/2019-02/prior-auth-2018.pdf. Published 2018. Accessed August 16, 2021.
17. Howell S, Yin PT, Robinson JC. Quantifying The Economic Burden Of Drug Utilization Management On Payers, Manufacturers, Physicians, And Patients. *Health Affairs.* 2021;40(8):1206-1214.

18. Zon RT, Frame JN, Neuss MN, et al. American Society of Clinical Oncology Policy Statement on Clinical Pathways in Oncology. *Journal of Oncology Practice.* 2016;12(3):261-266.